15% to 100%. In Italy only 9 molecules are reimbursed in retail pharmacy and other 10 are not reimbursed at all. In 63% cases ex-factory prices in Italy are higher than in France. The average price difference in price is 12 % with 50% of products sharing almost same price (less than 5% difference). No information is available in France on managed entry agreement, while it is publicly available in Italy. This prevents fair price comparison. **CONCLUSIONS:** OD are more available in Italy, but reimbursement is poorer than in France. Prices are slightly higher in Italy but France displays multiple confidential rebates making it impossible to compare net prices. In Italy the actual accessibility depends a lot on regional level unlike France.

PSY103

HEALTH TECHNOLOGY ASSESSMENT, PRICE AND REIMBURSEMENT REVIEW FOR ORPHAN DRUGS IN ITALY

Tavella F[1], Korchagina D[2], Rodrigues J[2], Rémuzat C[2]

[1]*Creativ-Ceutical, London, UK,* [2]*Creativ-Ceutical, Paris, France*

OBJECTIVES: In Italy drug Health Technology Assessment (HTA) is conducted by the Scientific Technical Commission of Italian Medicines Agency (AIFA) with further negotiation between the manufacturers and the AIFA's Pricing & Reimbursement Committee on price and reimbursement. After the decision is taken it is published in the official journal (Gazetta officale), the assessed drug is formally available for Italian patients next day. There does not exist a specific procedure for orphan drugs (OD), they are evaluated under the same conditions as drugs for common diseases. Pharmaco-economic studies are recommended for innovative drugs. The objective of the study is to review HTA decisions, prices and reimbursement of OD in Italy. **METHODS:** All OD assessed in Italy since 2000 were identified. Prices, reimbursement rates and decision details were extracted for each drug using Farmadati Italia database. **RESULTS:** Among 74 OD approved in Europe 66 molecules are officially available in Italy. It took 5-10 months from granting market authorization to final decision on pricing and reimbursement and publication in 'Gazetta officale'. The mean time was about 17 months shorter than for common drugs. Only 9 of available molecules are fully reimbursed in Italy, reimbursement of 30 drugs is restricted to hospital use, 10 are not reimbursed and 5 are in a special class waiting a decision on reimbursement rate. For other drugs information is not available. Post-marketing surveillance studies were requested for a half of indications, AIFA registries were reported in 25% of cases. Annual treatment ex-factory price of OD varies from € 2 500 to almost € 1 000 000. **CONCLUSIONS:** Almost 90% of approved OD are available in Italy. However, the actual situation often differs a lot from the official data as some drugs are unavailable from retail or hospital pharmacies. In addition, regional authorities contribute to inequity in access especially for "expensive drugs".

PSY104

HEALTH TECHNOLOGY ASSESSMENT, PRICE AND REIMBURSEMENT REVIEW FOR ORPHAN DRUGS IN FRANCE

Korchagina D[1], Rémuzat C[1], Rodrigues J[1], Kornfeld A[2], Toumi M[3]

[1]*Creativ-Ceutical, Paris, France,* [2]*Creativ Ceutical, Paris, France,* [3]*University of Marseille, Marseille, France*

OBJECTIVES: In France Orphan Drugs (OD) undergo the same Health Technology Assessment (HTA) procedure as other drugs. The evaluation is performed by the Transparency Committee (TC). Two scores are assigned and further used for pricing & reimbursement decision: drug's medical benefit (SMR) and improvement in medical benefit (ASMR). OD can be eligible to an accelerated procedure established for innovative products. The study aim is to analyse HTA decisions, prices and reimbursement of OD in France. **METHODS:** Exhaustive prices, reimbursement and HTA opinions were extracted using Transparency Committee Website, AMELI's national health insurance and Thériaque databases. **RESULTS:** Among 74 OD approved in Europe, 21 drugs are not available in France. 6 were recently assessed by TC but are not yet reimbursed, 14 were not assessed and one got a negative opinion. Reimbursement process took between 2 and 5 months after TC opinion. 88% of drugs were considered as bringing a substantial medical benefit. At the same time, more than a half of medicines were graded as providing a major (9%), significant (27%) or moderate (20%) improvement in actual benefit leading to opportunity for premium prices, 30% were rated minor improvement, and about 14% no improvement according to TC opinion. Annual treatment prices of OD varie from € 1,500 to almost € 1,000,000. **CONCLUSIONS:** About a third of approved ODs are not available in France. Most of them were authorized recently and are might become available after the HTA and pricing process. In comparison to non OD, the ASMR scoring is outstanding. Lack of alternative treatment and severity of the condition appear to be the drivers of high SMR-ASMR score.

PSY105

HTA STUDIES ON ORPHAN DRUGS BY REBRATSXMEMBERS

Souza KM[1], Gonçalves L[2]

[1]*Brazilian Ministry of Health, Brasília, Brazil,* [2]*Brazilian Ministry of Health, Brasília-DF, Brazil*

OBJECTIVES: In Brazil, the studies produced by members of the Brazilian Network for Health Technology Assessment (REBRATS) have contributed in a significant way in the process of management and incorporation of technologies in the Brazilian Public Health system (SUS). However, there is still a lack of a Pharmaceutical Assistance and HTA Policy for rare diseases and the evidence for orphan drugs are limited and lower. The coverage for these drugs, is frequently done through judicial orders, political and social pressure, with no support of evidence-based medicine. The objectives is to evaluate the production of HTA studies for orphan drugs made by REBRATS members. **METHODS:** Query to the REBRATS database and the internal production of the HTA coordination, prioritizing 6 major diseases: Gaucher disease, Fabry disease, Mucopolysaccharidosis Type I (MPS I); Mucopolysaccharidosis Type II (MPS II); Mucopolysaccharidosis Type VI (MPS VI); Paroxysmal Nocturnal Haemoglobinuria (PNH). **RESULTS:** Five HTA studies were found, which are: a Rapid Response for Gaucher disease; a study on Health Technology Management for Fabry disease; an Economic Evaluation for MPS I, II and VI; a Systematic Review for Mucopolysaccharidosis Type II; and one Rapid Review for PNH. For the evaluated Orphan Drugs, only the Eculizumabe for the treatment of PNH has no approved registration by the National Health Surveillance Agency (ANVISA). **CONCLUSIONS:** Considering its high cost, high judicial demand and limited availability of scientific evidence, orphan drugs represent a challenge for researchers and decision makers. Clinical benefit, disease severity, availability of therapeutic alternatives, ethical, political and social aspects should be considered. It is necessary to start a multidisciplinary reflection on the development of HTA models and policies regarding rare diseases and innovative treatments in the SUS, as well as fostering the primary researches in this field.

PSY106

TO WHAT EXTENT DO DISEASE AND TREATMENT CHARACTERISTICS INFLUENCE HTA-BASED RECOMMENDATIONS FOR A SAMPLE OF ORPHAN DRUGS IN THREE COUNTRIES, AND COULD THESE INDICATE WHETHER ORPHAN DRUGS HAVE A "SPECIAL STATUS"?

Nicod E

London School of Economics and Political Science, London, UK

Routine HTA methods may not adequately capture all the important considerations of a treatment's value and the impact of the condition on the patient given that evidence is often incomplete. This study aims to explore the influence of broader considerations of scientific and social value judgments on reimbursement decisions for a sample of orphan drugs.**OBJECTIVES:** To identify and compare the extent to which these broader considerations not captured by the incremental cost-effectiveness ratio (ICER) influenced HTA decision-making process in three countries; and, on this basis, explore whether orphan drugs have a "special status". **METHODS:** Countries included were England, Scotland and Sweden. Ten drug-indication pairs with EMA orphan designation and all appraised by NICE were selected. Publicly available HTA reports were coded using thematic analysis to systematically identify and compare these broader considerations across countries using an existing analytical framework. **RESULTS:** 108 different other considerations were identified and grouped into 15 clusters based on the information provided. The most common related to the nature of the disease, and considerations based on rarity or unmet need. 52% were one of the main reasons for the decision, and in some cases, were also a pivotal factor in accepting high and uncertain ICERs. Categorising these as social or scientific value judgments was done to identify areas where further elicitation of societal preferences, and where more consistency and transparency in their use are needed, respectively. Each of these was then compared to determine whether they pertained specifically to orphan drug or rare disease characteristics. **CONCLUSIONS:** Considerable variation was seen in the application of these broader considerations. Identifying these is a way forward to highlight areas where more research, or consistency and transparency are needed. Some of these other considerations may also favour orphan drugs, furthering the debate around whether orphan drugs deserve special status.

PSY107

WHY ARE THERE DIFFERENCES IN HTA RECOMMENDATIONS ACROSS COUNTRIES? A SYSTEMATIC COMPARISON OF HTA DECISION PROCESSES FOR A SAMPLE OF ORPHAN DRUGS IN FOUR COUNTRIES

Nicod E

London School of Economics and Political Science, London, UK

HTA reimbursement recommendations often result in different outcomes across countries despite the same evidence being appraised for a same technology. There is a need to understand the reasons for these differences. **OBJECTIVES:** To systematically compare HTA processes for a sample of orphan drugs across four countries (England, Scotland, Sweden, France): to identify the use and interpretation of the evidence appraised, and highlight differences across countries. **METHODS:** Ten orphan drug-indication pairs were selected and systematically compared using a previously validated framework. An exploratory sequential mixed methods design divided the research into two stages: (1) qualitative in-depth analysis of the decision-making processes; and (2) quantitative identification of agency-specific risk preferences and agreement levels across countries. **RESULTS:** Differences at each step of the decision-making process were identified. The same pivotal trials were appraised but with varying levels of detail in reporting the clinical outcomes, explaining some of the reasons for differing HTA recommendations. Agency-specific risk preferences were identified through correspondence analysis as drivers of these decisions, further explaining some of these differences. Poor to moderate agreement in the interpretation of the evidence was measured using Cohen's kappa scores. This reflected situations where the countries interpreted the same evidence differently and situations where differences in the handling of the same uncertainties were seen, including differences in the extent to which stakeholder input influenced a decision. **CONCLUSIONS:** This research systematically compared HTA processes in different countries, facilitating the understanding of these complex processes including how different HTA bodies conduct value assessments. It enabled to raise awareness around the reasons for differences across countries, and highlight areas for potential methodological improvements in HTA. Further application of this framework to other disease areas and countries is a way forward to improving the drivers of coverage decisions while better understanding the settings and limitations of HTA.

PSY108

TOP 20 ORPHAN DRUGS AVAILABILITY, PRICING AND REIMBURSEMENT IN SLOVAKIA: 2005-2012 REVIEW

Babela R[1], Uraz V[2], Babelova O[2], Slezakova Z[1]

[1]*St. Elizabeth University, BRATISLAVA, Slovak Republic,* [2]*St. Elizabeth University, Bratislava, Slovak Republic*

OBJECTIVES: Orphan drugs are highly priced and top 20 orphan drugs create almost 2,5% of total drug expenditure in Slovakia. We conducted 8 years review of government and literature sources to provide insight into pricing, reimbursement and availability situation surrounding top 20 orphan drugs in Slovakia from the health care payer perspective. **METHODS:** We provide analysis of official prices, reim-